Placental Mesenchymal Stem Cell Augmentation of Fetal Myelomeningocele Repair

Grant Award Details

Placental Mesenchymal Stem Cell Augmentation of Fetal Myelomeningocele Repair

Grant Type: Late Stage Preclinical Projects

Grant Number: CLIN1-11404

Investigator:

Name:	Diana Farmer
Institution:	University of California, Davis
Type:	PI

Disease Focus: Neurological Disorders, Spina Bifida

Human Stem Cell Use: Adult Stem Cell

Award Value: $5,666,077

Status: Pre-Active

Grant Application Details

Application Title: Placental Mesenchymal Stem Cell Augmentation of Fetal Myelomeningocele Repair

Public Abstract:

Therapeutic Candidate or Device

Allogeneic Placenta-derived Mesenchymal Stem Cells Seeded on Cook Biodesign® Dural Graft Extracellular Matrix (PMSC-ECM)

Indication

Myelomeningocele (MMC) -or Spina Bifida -diagnosed prenataly

Therapeutic Mechanism

Placenta-derived mesenchymal stem cells (PMSCs) act by a paracrine mechanism, secreting a variety of growth factors, cytokines, and extracellular vesicles. This secretory profile is unique to PMSCs and is responsible for protecting motor neurons from apoptosis, which occurs due to chemical and mechanical trauma when motor neurons are exposed to the intrauterine environment. PMSC treatment increases the density of motor neurons in the spinal cord, leading to improved motor function.

Unmet Medical Need

The current standard of care in utero surgery, while promising, still leaves 58% of patients unable to walk independently. There is an extraordinary need for a therapy that prevents or lessens the severity of the devastating and costly lifelong disabilities associated with the disease.

Project Objective

IND filing, Phase 1/2 trial start-up activities

Major Proposed Activities

- Manufacture product to supply the proposed studies and clinical trial
- Assess safety of the therapeutic PMSC product
- Assess efficacy using clinical-grade product

Statement of Benefit to California:

There is a high incidence of MMC in CA with 39.1% of the population being of Hispanic or Latino descent, a demographic that is affected by MMC at a disproportionately high rate. The cost to CA is approximately $532,000 per child, but for many, the cost may be several million dollars due to ongoing treatment. Indirect costs include pain and suffering, specialized childcare, and lost time of unpaid caregivers. A therapy for MMC would relieve the tremendous emotional and economic cost burden to CA.

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